```
=> d his

     (FILE 'HOME' ENTERED AT 16:32:38 ON 22 SEP 2008)

     FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 16:33:00 ON
     22 SEP 2008
L1          320 S (TREAT? OR REDUC? OR INHIBIT? OR AMELIORAT?)(7A)FABRY(W)DISEA
L2         5631 S FABRY(W)DISEASE
L3          310 S (GENE OR CDNA OR POLYNUCLEOTIDE OR NUCLEIC(W)ACID OR VECTOR O
L4           30 S L1 AND L3
L5          161 S L2(P)L3
L6           18 DUP REM L4 (12 DUPLICATES REMOVED)
L7           96 DUP REM L5 (65 DUPLICATES REMOVED)

=> d au ti so pi 1-18 l6

L6   ANSWER 1 OF 18  BIOSIS  COPYRIGHT (c) 2008 The Thomson Corporation  on STN
AU   Shimotori, Masaaki; Maruyama, Hiroki [Reprint Author]; Nakamura, Gen;
     Suyama, Takayuki; Sakamoto, Fumiko; Itoh, Masaaki; Miyabayashi, Shigeaki;
     Ohnishi, Takahiro; Sakai, Norio; Wataya-Kaneda, Mari; Kubota, Mitsuru;
     Takahashi, Toshiyuki; Mori, Tatsuhiko; Tamura, Katsuhiko; Kageyama,
     Shinji; Shio, Nobuo; Maeba, Teruhiko; Yahagi, Hirokazu; Tanaka, Motoko;
     Oka, Masayo; Sugiyama, Hitoshi; Sugawara, Toshiyuki; Mori, Noriko;
     Tsukamoto, Hiroko; Tamagaki, Keiichi; Tanda, Shuuji; Suzuki, Yuka;
     Shinonaga, Chiya; Miyazaki, Jun-Ichi; Ishii, Satoshi; Gejyo, Fumitake
TI   Novel mutations of the GLA gene in Japanese patients with Fabry disease
     and their functional characterization by active site specific chaperone.
SO   Human Mutation, (FEB 2008) Vol. 29, No. 2, pp. 331.
     ISSN: 1059-7794.

L6   ANSWER 2 OF 18     MEDLINE on STN                  DUPLICATE 1
AU   Nakamura Gen; Maruyama Hiroki; Ishii Satoshi; Shimotori Masaaki; Kameda
     Shigemi; Kono Toru; Miyazaki Jun-ichi; Kulkarni Ashok B; Gejyo Fumitake
TI   Naked plasmid DNA-based alpha-galactosidase
     A gene transfer partially reduces systemic accumulation of
     globotriaosylceramide in Fabry mice.
SO   Molecular biotechnology, (2008 Feb) Vol. 38, No. 2, pp. 109-19.
     Electronic Publication: 2007-10-13.
     Journal code: 9423533. ISSN: 1073-6085.

L6   ANSWER 3 OF 18  BIOSIS  COPYRIGHT (c) 2008 The Thomson Corporation  on STN
AU   Yoshimitsu, M.; Higuchi, K.; Ramsubir, S.; Nonaka, T.; Rasaiah, V. I.;
     Siatskas, C.; Liang, S. -B; Murray, G. J.; O Brady, R.; Medin, J. A.
     [Reprint Author]
TI   Efficient correction of Fabry mice and patient cells mediated by
     lentiviral transduction of hematopoietic stem/progenitor cells.
SO   Gene Therapy, (FEB 2007) Vol. 14, No. 3, pp. 256-265.
     ISSN: 0969-7128.

L6   ANSWER 4 OF 18  CAPLUS  COPYRIGHT 2008 ACS on STN
AU   Sirrs, Sandra M.; Clarke, Joe T. R.
TI   Agalsidase alfa therapy for Fabry disease
SO   Expert Review of Endocrinology & Metabolism (2007), 2(2), 147-154
     CODEN: EREMBI; ISSN: 1744-6651

L6   ANSWER 5 OF 18  BIOSIS  COPYRIGHT (c) 2008 The Thomson Corporation  on STN
AU   Bekri, Soumeya [Reprint Author]; Enica, Adrian; Ghafari, Thomas; Plaza,
     Gisele; Champenois, Isabelle; Choukroun, Gabriel; Unwin, Robert; Jaeger,
     Philippe
TI   Fabry disease in patients with end-stage renal failure: The potential
     benefits of screening.
```

SO Nephron Clinical Practice, (2005) Vol. 101, No. 1, pp. C33-C38.
ISSN: 1660-2110.

L6 ANSWER 6 OF 18 MEDLINE on STN DUPLICATE 2
AU Yoshimitsu Makoto; Sato Takeya; Tao Kesheng; Walia Jagdeep S; Rasaiah Vanessa I; Sleep Gillian T; Murray Gary J; Poeppl Armando G; Underwood John; West Lori; Brady Roscoe O; Medin Jeffrey A
TI Bioluminescent imaging of a marking transgene and correction of Fabry mice by neonatal injection of recombinant lentiviral vectors.
SO Proceedings of the National Academy of Sciences of the United States of America, (2004 Nov 30) Vol. 101, No. 48, pp. 16909-14. Electronic Publication: 2004-11-18.
Journal code: 7505876. ISSN: 0027-8424.

L6 ANSWER 7 OF 18 MEDLINE on STN DUPLICATE 3
AU Ziegler Robin J; Lonning Scott M; Armentano Donna; Li Chester; Souza David W; Cherry Maribeth; Ford Christine; Barbon Christine M; Desnick Robert J; Gao Guangping; Wilson James M; Peluso Richard; Godwin Simon; Carter Barrie J; Gregory Richard J; Wadsworth Samuel C; Cheng Seng H
TI AAV2 vector harboring a liver-restricted promoter facilitates sustained expression of therapeutic levels of alpha-galactosidase A and the induction of immune tolerance in Fabry mice.
SO Molecular therapy : the journal of the American Society of Gene Therapy, (2004 Feb) Vol. 9, No. 2, pp. 231-40.
Journal code: 100890581. ISSN: 1525-0016.

L6 ANSWER 8 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN
IN Treco, Douglas A.; Loveday, Kenneth; Borowski, Marianne
TI Treatment of α-galactosidase A deficiency
SO PCT Int. Appl., 94 pp.
CODEN: PIXXD2

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI WO 2003090695	A2	20031106	WO 2003-US13063	20030425
WO 2003090695	A3	20040506		
W: AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, OM, PH, PL, PT, RO, RU, SD, SE, SG, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, UZ, VN, YU, ZA, ZM, ZW				
RW: GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW, AM, AZ, BY, KG, KZ, MD, RU, TJ, TM, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR, BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG				
CA 2483270	A1	20031106	CA 2003-2483270	20030425
AU 2003228722	A1	20031110	AU 2003-228722	20030425
US 20040071686	A1	20040415	US 2003-423225	20030425
EP 1503788	A2	20050209	EP 2003-726489	20030425
R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO, MK, CY, AL, TR, BG, CZ, EE, HU, SK				
BR 2003009665	A	20050816	BR 2003-9665	20030425
JP 2005538941	T	20051222	JP 2003-587334	20030425
NZ 536361	A	20080530	NZ 2003-536361	20030425
US 20060177433	A1	20060810	US 2006-403618	20060413

L6 ANSWER 9 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN
IN Selden, Richard F.; Borowski, Marianne; Kinoshita, Carol M.; Treco, Douglas A.; Williams, Melanie D.; Schuetz, Thomas J.; Daniel, Peter F.
TI Purification of recombinant α-galactosidase A and its glycosylation modification for treatment of Fabry disease

and related therapy by targeted gene activation
SO U.S., 39 pp., Cont.-in-part of U. S. Ser. No. 928,881.
CODEN: USXXAM

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	US 6458574	B1	20021001	US 1999-266014	19990311
	WO 9811206	A2	19980319	WO 1997-US16603	19970912
	WO 9811206	A3	19980813		
	W: AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CU, CZ, DE, DK, EE, ES, FI, GB, GE, GH, HU, IL, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, UA, UG, US, UZ, VN, YU, ZW				
	RW: GH, KE, LS, MW, SD, SZ, UG, ZW, AT, BE, CH, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, ML, MR, NE, SN, TD, TG				
	US 6083725	A	20000704	US 1997-928881	19970912
	CA 2365923	A1	20000914	CA 2000-2365923	20000309
	WO 2000053730	A2	20000914	WO 2000-US6118	20000309
	WO 2000053730	A3	20010315		
	W: AE, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CR, CU, CZ, DE, DK, DM, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZW				
	RW: GH, GM, KE, LS, MW, SD, SL, SZ, TZ, UG, ZW, AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG				
	AU 2000035194	A	20000928	AU 2000-35194	20000309
	EP 1163349	A2	20011219	EP 2000-913825	20000309
	EP 1163349	B1	20080220		
	R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO, CY				
	CN 1354796	A	20020619	CN 2000-807312	20000309
	HU 2002000467	A2	20020629	HU 2002-467	20000309
	HU 2002000467	A3	20060628		
	JP 2002538183	T	20021112	JP 2000-603353	20000309
	NZ 514077	A	20040227	NZ 2000-514077	20000309
	RU 2248213	C2	20050320	RU 2001-127533	20000309
	EP 1820862	A2	20070822	EP 2006-25159	20000309
	EP 1820862	A3	20071031		
	R: AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LI, LU, MC, NL, PT, SE				
	AT 386808	T	20080315	AT 2000-913825	20000309
	ES 2300256	T3	20080616	ES 2000-913825	20000309
	CN 101219213	A	20080716	CN 2007-10148292	20000309
	NO 2001004415	A	20011112	NO 2001-4415	20010911
	MX 2001PA09222	A	20020604	MX 2001-PA9222	20010911
	AU 762400	B2	20030626	AU 2001-93403	20011123
	US 20030077806	A1	20030424	US 2002-165060	20020607
	US 20030113894	A1	20030619	US 2002-165968	20020610
	HK 1043386	A1	20080613	HK 2002-104366	20020611
	AU 2003220717	A1	20030814	AU 2003-220717	20030722
	AU 2003220717	B2	20071018		
	AU 2004242550	A1	20050127	AU 2004-242550	20041231
	AU 2004242550	B2	20080403		
	KR 2007090277	A	20070905	KR 2007-719031	20070820
	AU 2008200265	A1	20080207	AU 2008-200265	20080118
	AU 2008202567	A1	20080703	AU 2008-202567	20080611

L6 ANSWER 10 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN DUPLICATE 4

AU Ashley, Grace A.; Desnick, Robert J.; Gordon, Ronald E.; Gordon, Jon W.
TI High overexpression of the human α-galactosidase A gene driven by its promoter in transgenic mice: implications for the treatment of Fabry disease
SO Journal of Investigative Medicine (2002), 50(3), 185-192
CODEN: JINVFI; ISSN: 1081-5589

L6 ANSWER 11 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN
AU Germain, Dominique P.
TI Fabry disease (α-galactosidase A deficiency): new therapeutic prospects
SO Journal de la Societe de Biologie (2002), 196(2), 183-190
CODEN: JDSBFG; ISSN: 1295-0661

L6 ANSWER 12 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN
AU Germain, Dominique Paul
TI Fabry disease (α-galactosidase A deficiency): pathophysiology, clinical signs and genetics aspects
SO Journal de la Societe de Biologie (2002), 196(2), 161-173
CODEN: JDSBFG; ISSN: 1295-0661

L6 ANSWER 13 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN
AU Takiyama, Nobuaki
TI Retroviral mediated gene transfer to human CD34+ hematopoietic cells: gene therapy of Gaucher and Fabry diseases
SO Keio Igaku (2002), 79(1), T143-T152
CODEN: KEIGAS; ISSN: 0368-5179

L6 ANSWER 14 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN
IN Miyamura, Nobuhiro
TI Protein and cDNA sequences of truncated human .alpha.-galactosidase A and uses thereof in treatment of Fabry disease
SO U.S., 51 pp.
CODEN: USXXAM

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI US 6210666	B1	20010403	US 1998-176666	19981021

L6 ANSWER 15 OF 18 BIOSIS COPYRIGHT (c) 2008 The Thomson Corporation on STN
AU Jung, Sung-Chul; Han, Ina P.; Limaye, Advait; Xu, Ruian; Gelderman, Monique P.; Zerfas, Patricia; Tirumalai, Kamala; Murray, Gary J.; During, Matthew J.; Brady, Roscoe O.; Qasba, Pankaj [Reprint author]
TI Adeno-associated viral vector-mediated gene transfer results in long-term enzymatic and functional correction in multiple organs of Fabry mice.
SO Proceedings of the National Academy of Sciences of the United States of America, (February 27, 2001) Vol. 98, No. 5, pp. 2676-2681. print.
CODEN: PNASA6. ISSN: 0027-8424.

L6 ANSWER 16 OF 18 BIOSIS COPYRIGHT (c) 2008 The Thomson Corporation on STN
AU Ohshima, Toshio; Schiffmann, Raphael; Murray, Gary J.; Kopp, Jeffrey; Quirk, Janey M.; Stahl, Stefanie; Chan, Chi-Chao; Zerfas, Patricia; Tao-Cheng, Jung-Hwa; Ward, J. M.; Brady, Roscoe O.; Kulkarni, Ashok B. [Reprint author]
TI Aging accentuates and bone marrow transplantation ameliorates metabolic defects in Fabry disease mice.
SO Proceedings of the National Academy of Sciences of the United States of America, (May 25, 1999) Vol. 96, No. 11, pp. 6423-6427. print.
CODEN: PNASA6. ISSN: 0027-8424.

L6 ANSWER 17 OF 18 MEDLINE on STN DUPLICATE 5
AU Ziegler R J; Yew N S; Li C; Cherry M; Berthelette P; Romanczuk H; Ioannou Y A; Zeidner K M; Desnick R J; Cheng S H
TI Correction of enzymatic and lysosomal storage defects in Fabry mice by adenovirus-mediated gene transfer.
SO Human gene therapy, (1999 Jul 1) Vol. 10, No. 10, pp. 1667-82.
Journal code: 9008950. ISSN: 1043-0342.

L6 ANSWER 18 OF 18 CAPLUS COPYRIGHT 2008 ACS on STN
IN Desnick, Robert J.; Bishop, David F.; Ioannou, Yiannis A.
TI Biologically active human α-galactosidase A and its manufacture by expression of the cloned gene
SO PCT Int. Appl., 154 pp.
CODEN: PIXXD2

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI WO 9412628	A1	19940609	WO 1993-US11539	19931130
W: AU, BB, BG, BR, BY, CA, CZ, FI, HU, JP, KR, KZ, LK, LV, MG, MN, MW, NO, NZ, PL, RO, RU, SD, SK, UA, UZ				
RW: AT, BE, CH, DE, DK, ES, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, ML, MR, NE, SN, TD, TG				
US 5401650	A	19950328	US 1992-983451	19921130
AU 9456817	A	19940622	AU 1994-56817	19931130
AU 691795	B2	19980528		
EP 670896	A1	19950913	EP 1994-902448	19931130
EP 670896	B1	20020206		
EP 670896	B2	20050427		
R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IE, IT, LI, LU, MC, NL, PT, SE				
JP 08503615	T	19960423	JP 1994-513423	19931130
JP 4005629	B2	20071107		
AT 213020	T	20020215	AT 1994-902448	19931130

=>
=> d au ti so pi 50-70 l7

L7 ANSWER 50 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Ashley, Grace Ann
TI α-galactosidase a: mutation analysis in patients with fabry disease and expression and regulation in transgenic mice
SO (2001) 150 pp. Avail.: UMI, Order No. DA3006671
From: Diss. Abstr. Int., B 2001, 62(2), 661

L7 ANSWER 51 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
IN Miyamura, Nobuhiro
TI Protein and cDNA sequences of truncated human .alpha.-galactosidase A and uses thereof in treatment of Fabry disease
SO U.S., 51 pp.
CODEN: USXXAM

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI US 6210666	B1	20010403	US 1998-176666	19981021

L7 ANSWER 52 OF 96 MEDLINE on STN DUPLICATE 17
AU Qin G; Takenaka T; Telsch K; Kelley L; Howard T; Levade T; Deans R; Howard B H; Malech H L; Brady R O; Medin J A
TI Preselective gene therapy for Fabry disease.
SO Proceedings of the National Academy of Sciences of the United States of America, (2001 Mar 13) Vol. 98, No. 6, pp. 3428-33. Electronic

Publication: 2001-03-06.
Journal code: 7505876. ISSN: 0027-8424.

L7 ANSWER 53 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Itoh, Yoshiaki; Esaki, Takanori; Cook, Michelle; Qasba, Pankaj; Shimoji, Kazuaki; Alroy, Joseph; Brady, Roscoe O.; Sokoloff, Louis; Moore, David F.
TI Local and global cerebral blood flow and glucose utilization in the α-galactosidase A knockout mouse model of Fabry disease
SO Journal of Neurochemistry (2001), 79(6), 1217-1224
CODEN: JONRA9; ISSN: 0022-3042

L7 ANSWER 54 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Whybra, C.; Kampmann, Chr.; Willers, I.; Davies, J.; Winchester, B.; Kriegsmann, J.; Bruhl, K.; Gal, A.; Bunge, S.; Beck, M.
TI Anderson-Fabry disease: Clinical manifestations of disease in female heterozygotes
SO Journal of Inherited Metabolic Disease (2001), 24(7), 715-724
CODEN: JIMDDP; ISSN: 0141-8955

L7 ANSWER 55 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Konoshita, T.; Mutoh, H.; Yokoi, T.; Koni, I.; Miyamori, I.; Mabuchi, H.
TI A missense mutation, A156T, in the α-galactosidase A gene causes typical Fabry disease
SO Clinical Nephrology (2001), 55(3), 243-247
CODEN: CLNHBI; ISSN: 0301-0430

L7 ANSWER 56 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Germain, Dominique P.
TI A new phenotype of Fabry disease with intermediate severity between the classical form and the cardiac variant
SO Contributions to Nephrology (2001), 136(Rare Kidney Diseases), 234-240
CODEN: CNEPDD; ISSN: 0302-5144

L7 ANSWER 57 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Manni, E.; Fogli, A.; Baldinotti, F.; Rossi, S.; Funel, N.; Barachini, P.; Simi, P.
TI Nonsense mutation in exon 2 of the α-galactosidase A gene in a patient with Fabry disease
SO Contributions to Nephrology (2001), 136(Rare Kidney Diseases), 223-228
CODEN: CNEPDD; ISSN: 0302-5144

L7 ANSWER 58 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Desnick, Robert J.; Wasserstein, Melissa P.; Banikazemi, Maryam
TI Fabry disease (α-galactosidase A deficiency): renal involvement and enzyme replacement therapy
SO Contributions to Nephrology (2001), 136(Rare Kidney Diseases), 174-192
CODEN: CNEPDD; ISSN: 0302-5144

L7 ANSWER 59 OF 96 MEDLINE on STN DUPLICATE 18
AU Yew N S; Przybylska M; Ziegler R J; Liu D; Cheng S H
TI High and sustained transgene expression in vivo from plasmid vectors containing a hybrid ubiquitin promoter.
SO Molecular therapy : the journal of the American Society of Gene Therapy, (2001 Jul) Vol. 4, No. 1, pp. 75-82.
Journal code: 100890581. ISSN: 1525-0016.

L7 ANSWER 60 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
IN Selden, Richard F.; Borowski, Marianne; Gillispie, Frances P.; Kinoshita, Carol M.; Treco, Douglas A.; Williams, Melanie D.
TI Gene and enzyme replacement therapy for α-galactosidase A deficiency
SO U.S., 32 pp.

CODEN: USXXAM

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	US 6083725	A	20000704	US 1997-928881	19970912
	US 6458574	B1	20021001	US 1999-266014	19990311
	US 6566099	B1	20030520	US 2000-491759	20000127
	US 6395884	B1	20020528	US 2000-543921	20000406
	AU 762400	B2	20030626	AU 2001-93403	20011123
	US 20030077806	A1	20030424	US 2002-165060	20020607
	US 20030113894	A1	20030619	US 2002-165968	20020610
	US 20030152560	A1	20030814	US 2002-318905	20021212
	US 7122354	B2	20061017		
	AU 2003220717	A1	20030814	AU 2003-220717	20030722
	AU 2003220717	B2	20071018		
	AU 2004242550	A1	20050127	AU 2004-242550	20041231
	AU 2004242550	B2	20080403		
	AU 2008200265	A1	20080207	AU 2008-200265	20080118
	AU 2008202567	A1	20080703	AU 2008-202567	20080611

L7 ANSWER 61 OF 96 MEDLINE on STN DUPLICATE 19
AU Ashton-Prolla P; Tong B; Shabbeer J; Astrin K H; Eng C M; Desnick R J
TI Fabry disease: twenty-two novel mutations in the alpha-galactosidase A gene and genotype/phenotype correlations in severely and mildly affected hemizygotes and heterozygotes.
SO Journal of investigative medicine : the official publication of the American Federation for Clinical Research, (2000 Jul) Vol. 48, No. 4, pp. 227-35.
Journal code: 9501229. ISSN: 1081-5589.

L7 ANSWER 62 OF 96 MEDLINE on STN DUPLICATE 20
AU Ziegler R J; Yew N S; Li C; Cherry M; Berthelette P; Romanczuk H; Ioannou Y A; Zeidner K M; Desnick R J; Cheng S H
TI Correction of enzymatic and lysosomal storage defects in Fabry mice by adenovirus-mediated gene transfer.
SO Human gene therapy, (1999 Jul 1) Vol. 10, No. 10, pp. 1667-82.
Journal code: 9008950. ISSN: 1043-0342.

L7 ANSWER 63 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Takenaka, Toshihiro; Hendrickson, Chad S.; Tworek, David M.; Tudor, Matthew; Schiffmann, Raphael; Brady, Roscoe O.; Medin, Jeffrey A.
TI Enzymatic and functional correction along with long-term enzyme secretion from transduced bone marrow hematopoietic stem/progenitor and stromal cells derived from patients with Fabry disease
SO Experimental Hematology (New York) (1999), 27(7), 1149-1159
CODEN: EXHMA6; ISSN: 0301-472X

L7 ANSWER 64 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Germain, Dominique P.; Poenaru, Livia
TI Fabry Disease: identification of novel alpha-galactosidase A mutations and molecular carrier detection by use of fluorescent chemical cleavage of mismatches
SO Biochemical and Biophysical Research Communications (1999), 257(3), 708-713
CODEN: BBRCA9; ISSN: 0006-291X

L7 ANSWER 65 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Beyeri, E. M.; Kopishinskaya, S. V.; Van Amstel, Ploos, J. K.; Tsvetkova, I. V.
TI Mutations of α-galactosidase A gene in two unusual cases of Fabry disease
SO Voprosy Meditsinskoi Khimii (1999), 45(4), 346-349

CODEN: VMDKAM; ISSN: 0042-8809

L7 ANSWER 66 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
IN Selden, Richard F.; Borowski, Marianne; Gillespie, Frances P.; Kinoshita, Carol M.; Treco, Douglas A.; Williams, Melanie D.
TI Gene and enzyme replacement therapy for α-galactosidase A deficiency
SO PCT Int. Appl., 78 pp.
CODEN: PIXXD2

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 9811206	A2	19980319	WO 1997-US16603	19970912
	WO 9811206	A3	19980813		
	W: AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CU, CZ, DE, DK, EE, ES, FI, GB, GE, GH, HU, IL, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, UA, UG, US, UZ, VN, YU, ZW				
	RW: GH, KE, LS, MW, SD, SZ, UG, ZW, AT, BE, CH, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, ML, MR, NE, SN, TD, TG				
	CA 2265464	A1	19980319	CA 1997-2265464	19970912
	CA 2265464	C	20070626		
	AU 9744244	A	19980402	AU 1997-44244	19970912
	EP 935651	A2	19990818	EP 1997-942567	19970912
	EP 935651	B1	20041229		
	R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, FI				
	CN 1230220	A	19990929	CN 1997-197909	19970912
	NZ 334721	A	20010126	NZ 1997-334721	19970912
	JP 2001504324	T	20010403	JP 1998-514004	19970912
	JP 4001925	B2	20071031		
	HU 9904666	A3	20010928	HU 1999-4666	19970912
	RU 2179034	C2	20020210	RU 1999-107287	19970912
	NZ 506214	A	20021126	NZ 1997-506214	19970912
	AT 286120	T	20050115	AT 1997-942567	19970912
	EP 1538202	A2	20050608	EP 2004-30107	19970912
	EP 1538202	A3	20070530		
	R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, FI				
	ES 2234032	T3	20050616	ES 1997-942567	19970912
	PL 190041	B1	20051031	PL 1997-332188	19970912
	TW 585919	B	20040501	TW 1997-86113342	19970913
	US 6458574	B1	20021001	US 1999-266014	19990311
	NO 9901225	A	19990510	NO 1999-1225	19990312
	HK 1022173	A1	20070119	HK 2000-101063	20000223
	AU 762400	B2	20030626	AU 2001-93403	20011123
	US 20030077806	A1	20030424	US 2002-165060	20020607
	US 20030113894	A1	20030619	US 2002-165968	20020610
	AU 2003220717	A1	20030814	AU 2003-220717	20030722
	AU 2003220717	B2	20071018		
	AU 2004242550	A1	20050127	AU 2004-242550	20041231
	AU 2004242550	B2	20080403		
	JP 2007000145	A	20070111	JP 2006-195854	20060718
	JP 2007289201	A	20071108	JP 2007-155943	20070613
	AU 2008200265	A1	20080207	AU 2008-200265	20080118
	AU 2008202567	A1	20080703	AU 2008-202567	20080611

L7 ANSWER 67 OF 96 MEDLINE on STN DUPLICATE 21
AU Sugimoto Y; Tsuruo T
TI In vivo drug-selectable markers in gene therapy.
SO Leukemia : official journal of the Leukemia Society of America, Leukemia

Research Fund, U.K, (1997 Apr) Vol. 11 Suppl 3, pp. 552-6.
Journal code: 8704895. ISSN: 0887-6924.

L7 ANSWER 68 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Desnick, Robert J.; Eng, Christine M.
TI Fabry disease: α-galactosidase A deficiency
SO Molecular and Genetic Basis of Neurological Disease (2nd Edition) (1997), 443-452. Editor(s): Rosenberg, Roger N. Publisher: Butterworth-Heinemann, Boston, Mass.
CODEN: 64KBAL

L7 ANSWER 69 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Eng, Christine M.; Ashley, Grace A.; Burgert, Tania S.; Enriquez, Annette L.; D'souza, Marcus; Desnick, Robert J.
TI Fabry disease: thirty-five mutations in the α-galactosidase A gene in patients with classic and variant phenotypes
SO Molecular Medicine (New York) (1997), 3(3), 174-182
CODEN: MOMEF3; ISSN: 1076-1551

L7 ANSWER 70 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Germain, Dominique; Biasotto, Michel; Tosi, Mario; Meo, Tommaso; Kahn, Axel; Poenaru, Livia
TI Fluorescence-assisted mismatch analysis (FAMA) for exhaustive screening of the α-galactosidase A gene and detection of carriers in Fabry disease
SO Human Genetics (1996), 98(6), 719-726
CODEN: HUGEDQ; ISSN: 0340-6717

=>
=> d au ti so pi 30-49 l7

L7 ANSWER 30 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Froissart, Roseline; Guffon, Nathalie; Vanier, Marie T.; Desnick, Robert J.; Maire, Irene
TI Fabry disease: D313Y is an α-galactosidase A sequence variant that causes pseudodeficient activity in plasma
SO Molecular Genetics and Metabolism (2003), 80(3), 307-314
CODEN: MGMEFF; ISSN: 1096-7192

L7 ANSWER 31 OF 96 MEDLINE on STN DUPLICATE 10
AU Yasuda Makiko; Shabbeer Junaid; Osawa Makiko; Desnick Robert J
TI Fabry disease: novel alpha-galactosidase A 3'-terminal mutations result in multiple transcripts due to aberrant 3'-end formation.
SO American journal of human genetics, (2003 Jul) Vol. 73, No. 1, pp. 162-73. Electronic Publication: 2003-06-06.
Journal code: 0370475. ISSN: 0002-9297.

L7 ANSWER 32 OF 96 BIOSIS COPYRIGHT (c) 2008 The Thomson Corporation on STN
AU Schaefer, E. [Reprint Author]; Whybra, C.; Widmer, U.; Osterziel, K. J.; Das, A.; Deegan, P.; Beck, M.; Gal, A. [Reprint Author]
TI Analysis of the gene for alpha-galactosidase A in Fabry disease: First-year experience.
SO Acta Paediatrica, (December 2003) Vol. 92, No. Supplement 443, pp. 108. print.
Meeting Info.: 3rd International Symposium on Lysosomal Diseases: Pathophysiology and Therapy. Santiago de Compostela, Spain. May, 2003.
ISSN: 0803-5253.

L7 ANSWER 33 OF 96 BIOSIS COPYRIGHT (c) 2008 The Thomson Corporation on

STN
AU Schaefer, E. [Reprint Author]; Gal, A. [Reprint Author]; Fabry Outcome Survey (FOS) Centres [Reprint Author]
TI Mutation spectrum of the gene for alpha-galactosidase A in Fabry disease.
SO Acta Paediatrica, (December 2003) Vol. 92, No. Supplement 443, pp. 107. print.
Meeting Info.: 3rd International Symposium on Lysosomal Diseases: Pathophysiology and Therapy. Santiago de Compostela, Spain. May, 2003.
ISSN: 0803-5253.

L7 ANSWER 34 OF 96 MEDLINE on STN DUPLICATE 11
AU Palla A; Widmer U; Straumann D
TI Head-impulse testing in Fabry disease--vestibular function in male and female patients.
SO Acta paediatrica (Oslo, Norway : 1992). Supplement, (2003 Dec) Vol. 92, No. 443, pp. 38-42; discussion 27.
Journal code: 9315043. ISSN: 0803-5326.

L7 ANSWER 35 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
IN Yew, Nelson
TI Gene therapy vectors containing hybrid ubiquitin promoters with high and persistent expression in target tissues
SO PCT Int. Appl., 29 pp.
CODEN: PIXXD2

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI WO 2002024932	A2	20020328	WO 2001-US28870	20010913
WO 2002024932	A3	20020815		
W: AU, CA, JP				
RW: AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, TR				
CA 2423082	A1	20020328	CA 2001-2423082	20010913
AU 2001090984	A	20020402	AU 2001-90984	20010913
US 20020090719	A1	20020711	US 2001-952152	20010913
US 6667174	B2	20031223		
EP 1319082	A2	20030618	EP 2001-971050	20010913
EP 1319082	B1	20051116		
R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, FI, CY, TR				
JP 2004516016	T	20040603	JP 2002-529524	20010913
AT 310096	T	20051215	AT 2001-971050	20010913
EP 1624067	A2	20060208	EP 2005-24580	20010913
EP 1624067	A3	20060315		
R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, FI, CY, TR				
ES 2252293	T3	20060516	ES 2001-971050	20010913
US 20070003521	A1	20070104	US 2003-661369	20030911

L7 ANSWER 36 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
IN Selden, Richard F.; Borowski, Marianne; Kinoshita, Carol M.; Treco, Douglas A.; Williams, Melanie D.; Schuetz, Thomas J.; Daniel, Peter F.
TI Purification of recombinant α-galactosidase A and its glycosylation modification for treatment of Fabry disease and related therapy by targeted gene activation
SO U.S., 39 pp., Cont.-in-part of U. S. Ser. No. 928,881.
CODEN: USXXAM

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI US 6458574	B1	20021001	US 1999-266014	19990311
WO 9811206	A2	19980319	WO 1997-US16603	19970912

WO 9811206 A3 19980813
W: AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CU, CZ, DE, DK, EE, ES, FI, GB, GE, GH, HU, IL, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, UA, UG, US, UZ, VN, YU, ZW
RW: GH, KE, LS, MW, SD, SZ, UG, ZW, AT, BE, CH, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, ML, MR, NE, SN, TD, TG
US 6083725 A 20000704 US 1997-928881 19970912
CA 2365923 A1 20000914 CA 2000-2365923 20000309
WO 2000053730 A2 20000914 WO 2000-US6118 20000309
WO 2000053730 A3 20010315
W: AE, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CR, CU, CZ, DE, DK, DM, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZW
RW: GH, GM, KE, LS, MW, SD, SL, SZ, TZ, UG, ZW, AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG
AU 2000035194 A 20000928 AU 2000-35194 20000309
EP 1163349 A2 20011219 EP 2000-913825 20000309
EP 1163349 B1 20080220
R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO, CY
CN 1354796 A 20020619 CN 2000-807312 20000309
HU 2002000467 A2 20020629 HU 2002-467 20000309
HU 2002000467 A3 20060628
JP 2002538183 T 20021112 JP 2000-603353 20000309
NZ 514077 A 20040227 NZ 2000-514077 20000309
RU 2248213 C2 20050320 RU 2001-127533 20000309
EP 1820862 A2 20070822 EP 2006-25159 20000309
EP 1820862 A3 20071031
R: AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LI, LU, MC, NL, PT, SE
AT 386808 T 20080315 AT 2000-913825 20000309
ES 2300256 T3 20080616 ES 2000-913825 20000309
CN 101219213 A 20080716 CN 2007-10148292 20000309
NO 2001004415 A 20011112 NO 2001-4415 20010911
MX 2001PA09222 A 20020604 MX 2001-PA9222 20010911
AU 762400 B2 20030626 AU 2001-93403 20011123
US 20030077806 A1 20030424 US 2002-165060 20020607
US 20030113894 A1 20030619 US 2002-165968 20020610
HK 1043386 A1 20080613 HK 2002-104366 20020611
AU 2003220717 A1 20030814 AU 2003-220717 20030722
AU 2003220717 B2 20071018
AU 2004242550 A1 20050127 AU 2004-242550 20041231
AU 2004242550 B2 20080403
KR 2007090277 A 20070905 KR 2007-719031 20070820
AU 2008200265 A1 20080207 AU 2008-200265 20080118
AU 2008202567 A1 20080703 AU 2008-202567 20080611

L7 ANSWER 37 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Takahashi, Hiroshi; Hirai, Yukihiko; Migita, Makoto; Seino, Yoshihiko; Fukuda, Yuh; Sakuraba, Hitoshi; Kase, Ryoichi; Kobayashi, Toshihide; Hashimoto, Yasuhiro; Shimada, Takashi
TI Long-term systemic therapy of Fabry disease in a knockout mouse by adeno-associated virus-mediated muscle-directed gene transfer
SO Proceedings of the National Academy of Sciences of the United States of America (2002), 99(21), 13777-13782

CODEN: PNASA6; ISSN: 0027-8424

L7 ANSWER 38 OF 96 MEDLINE on STN DUPLICATE 12
AU Germain Dominique P
TI Fabry disease: recent advances in enzyme replacement therapy.
SO Expert opinion on investigational drugs, (2002 Oct) Vol. 11, No. 10, pp. 1467-76. Ref: 55
Journal code: 9434197. ISSN: 1354-3784.

L7 ANSWER 39 OF 96 MEDLINE on STN DUPLICATE 13
AU Li Chester; Ziegler Robin J; Cherry Maribeth; Lukason Michael; Desnick Robert J; Yew Nelson S; Cheng Seng H
TI Adenovirus-transduced lung as a portal for delivering alpha-galactosidase A into systemic circulation for Fabry disease.
SO Molecular therapy : the journal of the American Society of Gene Therapy, (2002 Jun) Vol. 5, No. 6, pp. 745-54.
Journal code: 100890581. ISSN: 1525-0016.

L7 ANSWER 40 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Kimura, K.; Sato-Matsumura, K. C.; Nakamura, H.; Onodera, Y.; Morita, K.; Enami, N.; Shougase, T.; Ohsaki, T.; Kato, M.; Takahashi, T.; Yamaguchi, Y.; Shimizu, H.
TI A novel A97P amino acid substitution in α-galactosidase A leads to a classical Fabry disease with cardiac manifestations
SO British Journal of Dermatology (2002), 147(3), 545-548
CODEN: BJDEAZ; ISSN: 0007-0963

L7 ANSWER 41 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Germain, Dominique P.; Shabbeer, Junaid; Cotigny, Sylvie; Desnick, Robert J.
TI Fabry disease: Twenty novel α-galactosidase A mutations and genotype-phenotype correlations in classical and variant phenotypes
SO Molecular Medicine (Baltimore, MD, United States) (2002), 8(6), 306-312
CODEN: MOMEF3; ISSN: 1076-1551

L7 ANSWER 42 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Ashley, Grace A.; Desnick, Robert J.; Gordon, Ronald E.; Gordon, Jon W.
TI High overexpression of the human α-galactosidase A gene driven by its promoter in transgenic mice: implications for the treatment of Fabry disease
SO Journal of Investigative Medicine (2002), 50(3), 185-192
CODEN: JINVFI; ISSN: 1081-5589

L7 ANSWER 43 OF 96 MEDLINE on STN DUPLICATE 14
AU Germain Dominique P
TI [Fabry's disease (alpha-galactosidase-A deficiency): recent therapeutic innovations].
Maladie de Fabry (deficit en alpha-galactosidase A): innovations therapeutiques recentes.
SO Journal de la Societe de biologie, (2002) Vol. 196, No. 2, pp. 183-90. Ref: 41
Journal code: 100890617. ISSN: 1295-0661.

L7 ANSWER 44 OF 96 MEDLINE on STN DUPLICATE 15
AU Germain Dominique Paul
TI [Fabry's disease (alpha-galactosidase-A deficiency): physiopathology, clinical signs, and genetic aspects].
Maladie de Fabry (deficit en alpha-galactosidase A): physiopathologie, signes cliniques et aspects genetiques.
SO Journal de la Societe de biologie, (2002) Vol. 196, No. 2, pp. 161-73. Ref: 88

Journal code: 100890617. ISSN: 1295-0661.

L7 ANSWER 45 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Takiyama, Nobuaki
TI Retroviral mediated gene transfer to human CD34+ hematopoietic cells: gene therapy of Gaucher and Fabry diseases
SO Keio Igaku (2002), 79(1), T143-T152
CODEN: KEIGAS; ISSN: 0368-5179

L7 ANSWER 46 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Pastores, Gregory M.; Lien, Yeong-Hau H.
TI Biochemical and molecular genetic basis of Fabry disease
SO Journal of the American Society of Nephrology (2002), 13(6, Suppl. 2), S130-S133
CODEN: JASNEU; ISSN: 1046-6673

L7 ANSWER 47 OF 96 BIOSIS COPYRIGHT (c) 2008 The Thomson Corporation on STN DUPLICATE 16
AU Lien, Yeong-Hau H. [Reprint author]; Campbell, Paula D. [Reprint author]; Whitehair, Orlantha [Reprint author]; Lai, Li-Wen [Reprint author]
TI Ultraviolet irradiation enhances chimeric RNA/DNA oligonucleotide-medicated gene correction of alpha-galactosidase A in cultured fibroblasts from Fabry disease patient.
SO Journal of the American Society of Nephrology, (September, 2002) Vol. 13, No. Program and Abstracts Issue, pp. 128A. print.
Meeting Info.: Meeting of the American Society of Nephrology. Philadelphia, PA, USA. October 30-November 04, 2002. American Society of Nephrology.
CODEN: JASNEU. ISSN: 1046-6673.

L7 ANSWER 48 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Mutoh, Hisao
TI Fabry disease in patients with end-stage renal disease
SO Kanazawa Daigaku Juzen Igakkai Zasshi (2002), 111(1), 35-43
CODEN: JUZIAG; ISSN: 0022-7226

L7 ANSWER 49 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Shabbeer, Junaid; Yasuda, Makiko; Luca, Edlira; Desnick, Robert J.
TI Fabry disease: 45 novel mutations in the α-galactosidase A gene causing the classical phenotype
SO Molecular Genetics and Metabolism (2002), 76(1), 23-30
CODEN: MGMEFF; ISSN: 1096-7192

=> d au ti so pi 20-29 l7

L7 ANSWER 20 OF 96 LIFESCI COPYRIGHT 2008 CSA on STN
AU SchAefer, Ellen; Baron, Karin; Widmer, Urs; Deegan, Patrick; Neumann, Hartmut P.H.; Sunder-Plassmann, Gere; Johansson, Jan-Ove; Whybra, Catharina; Ries, Markus; Pastores, Gregory M.; Mehta, Atul; Beck, Michael; Gal, Andreas
TI Thirty-Four Novel Mutations of the GLA Gene in 121 Patients with Fabry Disease
SO Human Mutation [Hum. Mutat.], (20050000) vol. 25, no. 4, pp. 412-412. ISSN: 1059-7794.

L7 ANSWER 21 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Shabbeer, Junaid; Robinson, Misi; Desnick, Robert J.
TI Detection of α-galactosidase A mutations causing Fabry disease by denaturing high performance liquid chromatography

SO Human Mutation (2005), 25(3), 299-305
CODEN: HUMUE3; ISSN: 1059-7794

L7 ANSWER 22 OF 96 MEDLINE on STN DUPLICATE 5
AU Schaefer E; Mehta A; Gal A
TI Genotype and phenotype in Fabry disease: analysis of the Fabry Outcome Survey.
SO Acta paediatrica (Oslo, Norway : 1992). Supplement, (2005 Mar) Vol. 94, No. 447, pp. 87-92; discussion 79. Ref: 15
Journal code: 9315043. ISSN: 0803-5326.

L7 ANSWER 23 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
IN Fan, Jian-Qiang
TI Combination therapy for treating protein deficiencies
SO PCT Int. Appl., 40 pp.
CODEN: PIXXD2

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI WO 2004074450	A2	20040902	WO 2004-US4909	20040218
WO 2004074450	A3	20050825		
W: AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI				
RW: BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR, BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG				
US 20040219132	A1	20041104	US 2004-781356	20040217
CA 2516304	A1	20040902	CA 2004-2516304	20040218
EP 1594514	A2	20051116	EP 2004-712428	20040218
R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO, MK, CY, AL, TR, BG, CZ, EE, HU, SK				
BR 2004007648	A	20060221	BR 2004-7648	20040218
CN 1750834	A	20060322	CN 2004-80004467	20040218
JP 2006517980	T	20060803	JP 2006-503707	20040218
MX 2005PA07821	A	20060407	MX 2005-PA7821	20050722
IN 2005DN04230	A	20070427	IN 2005-DN4230	20050919

L7 ANSWER 24 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
IN Cheng, Seng H.; Meeker, David
TI Combined enzyme replacement, gene therapy and small molecule therapy for lysosomal storage diseases
SO U.S. Pat. Appl. Publ., 35 pp., Cont.-in-part of U.S. Ser. No. 884,526.
CODEN: USXXCO

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI US 20040204379	A1	20041014	US 2004-758773	20040116
US 20020095135	A1	20020718	US 2001-884526	20010619
US 20070280925	A1	20071206	US 2007-762689	20070613

L7 ANSWER 25 OF 96 MEDLINE on STN DUPLICATE 6
AU Yoshimitsu Makoto; Sato Takeya; Tao Kesheng; Walia Jagdeep S; Rasaiah Vanessa I; Sleep Gillian T; Murray Gary J; Poeppl Armando G; Underwood John; West Lori; Brady Roscoe O; Medin Jeffrey A
TI Bioluminescent imaging of a marking transgene and correction of Fabry mice by neonatal injection of recombinant lentiviral vectors.
SO Proceedings of the National Academy of Sciences of the United States of America, (2004 Nov 30) Vol. 101, No. 48, pp. 16909-14. Electronic Publication: 2004-11-18.
Journal code: 7505876. ISSN: 0027-8424.

L7 ANSWER 26 OF 96 CAPLUS COPYRIGHT 2008 ACS on STN
AU Kotanko, Peter; Kramar, Reinhard; Devrnja, Danijela; Paschke, Eduard; Voigtlaender, Till; Auinger, Martin; Demmelbauer, Klaus; Lorenz, Matthias; Hauser, Anna-Christine; Kofler, Hans-Joerg; Lhotta, Karl; Neyer, Ulrich; Pronai, Wolfgang; Wallner, Manfred; Wieser, Clemens; Wiesholzer, Martin; Zodl, Herbert; Foedinger, Manuela; Sunder-Plassmann, Gere
TI Results of a Nationwide Screening for Anderson-Fabry Disease among Dialysis Patients
SO Journal of the American Society of Nephrology (2004), 15(5), 1323-1329
CODEN: JASNEU; ISSN: 1046-6673

L7 ANSWER 27 OF 96 MEDLINE on STN DUPLICATE 7
AU Cho Monique E; Kopp Jeffrey B
TI Fabry disease in the era of enzyme replacement therapy: a renal perspective.
SO Pediatric nephrology (Berlin, Germany), (2004 Jun) Vol. 19, No. 6, pp. 583-93. Electronic Publication: 2004-04-03. Ref: 53
Journal code: 8708728. ISSN: 0931-041X.

L7 ANSWER 28 OF 96 MEDLINE on STN DUPLICATE 8
AU Ziegler Robin J; Lonning Scott M; Armentano Donna; Li Chester; Souza David W; Cherry Maribeth; Ford Christine; Barbon Christine M; Desnick Robert J; Gao Guangping; Wilson James M; Peluso Richard; Godwin Simon; Carter Barrie J; Gregory Richard J; Wadsworth Samuel C; Cheng Seng H
TI AAV2 vector harboring a liver-restricted promoter facilitates sustained expression of therapeutic levels of alpha-galactosidase A and the induction of immune tolerance in Fabry mice.
SO Molecular therapy : the journal of the American Society of Gene Therapy, (2004 Feb) Vol. 9, No. 2, pp. 231-40.
Journal code: 100890581. ISSN: 1525-0016.

L7 ANSWER 29 OF 96 MEDLINE on STN DUPLICATE 9
AU Yasuda Makiko; Shabbeer Junaid; Benson Stacy D; Maire Irene; Burnett Roger M; Desnick Robert J
TI Fabry disease: characterization of alpha-galactosidase A double mutations and the D313Y plasma enzyme pseudodeficiency allele.
SO Human mutation, (2003 Dec) Vol. 22, No. 6, pp. 486-92.
Journal code: 9215429. E-ISSN: 1098-1004.

=> s prothrombin(3a)enhancer
L8 32 PROTHROMBIN(3A) ENHANCER

=> d his

(FILE 'HOME' ENTERED AT 16:32:38 ON 22 SEP 2008)

FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 16:33:00 ON 22 SEP 2008
L1 320 S (TREAT? OR REDUC? OR INHIBIT? OR AMELIORAT?)(7A)FABRY(W)DISEA
L2 5631 S FABRY(W)DISEASE
L3 310 S (GENE OR CDNA OR POLYNUCLEOTIDE OR NUCLEIC(W)ACID OR VECTOR O
L4 30 S L1 AND L3
L5 161 S L2(P)L3
L6 18 DUP REM L4 (12 DUPLICATES REMOVED)
L7 96 DUP REM L5 (65 DUPLICATES REMOVED)
L8 32 S PROTHROMBIN(3A)ENHANCER

=> s l2 and l8
L9 1 L2 AND L8

```
=> s gene(w)therapy
L10     173348 GENE(W) THERAPY

=> s l8 and l10
L11         10 L8 AND L10

=> dup rem l11
PROCESSING COMPLETED FOR L11
L12          4 DUP REM L11 (6 DUPLICATES REMOVED)

=> d his

     (FILE 'HOME' ENTERED AT 16:32:38 ON 22 SEP 2008)

     FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 16:33:00 ON
     22 SEP 2008
L1          320 S (TREAT? OR REDUC? OR INHIBIT? OR AMELIORAT?)(7A)FABRY(W)DISEA
L2         5631 S FABRY(W)DISEASE
L3          310 S (GENE OR CDNA OR POLYNUCLEOTIDE OR NUCLEIC(W)ACID OR VECTOR O
L4           30 S L1 AND L3
L5          161 S L2(P)L3
L6           18 DUP REM L4 (12 DUPLICATES REMOVED)
L7           96 DUP REM L5 (65 DUPLICATES REMOVED)
L8           32 S PROTHROMBIN(3A)ENHANCER
L9            1 S L2 AND L8
L10      173348 S GENE(W)THERAPY
L11          10 S L8 AND L10
L12           4 DUP REM L11 (6 DUPLICATES REMOVED)

=> d bib ab l9

L9   ANSWER 1 OF 1  CAPLUS  COPYRIGHT 2008 ACS on STN
AN   2004:857159  CAPLUS
DN   141:325745
TI   Combined enzyme replacement, gene therapy and small molecule therapy for
     lysosomal storage diseases
IN   Cheng, Seng H.; Meeker, David
PA   USA
SO   U.S. Pat. Appl. Publ., 35 pp., Cont.-in-part of U.S. Ser. No. 884,526.
     CODEN: USXXCO
DT   Patent
LA   English
FAN.CNT 2
     PATENT NO.          KIND   DATE        APPLICATION NO.         DATE
     ---------------     ----   --------    --------------------    --------
PI   US 20040204379       A1    20041014    US 2004-758773          20040116
     US 20020095135       A1    20020718    US 2001-884526          20010619
     US 20070280925       A1    20071206    US 2007-762689          20070613
PRAI US 2000-212377P      P     20000619
     US 2001-884526       A2    20010619
AB   This invention provides various combinations of enzyme replacement
     therapy, gene therapy, and small mol. therapy for the treatment of
     lysosomal storage diseases.  Thus, in a mouse Fabry
     disease model, substrate deprivation therapy with
     deoxynorjirimycin derivative AMP-DNJ and D-threo-1-(3',4'-
     methylenedioxy)phenyl-2-palmitoylamino-3-pyrrolidino-1-propanol reduced
     reaccumulation of globotriaosylceramide GL3 following its reduction by enzyme
     replacement therapy with α-galactosidase A.  Addnl., adeno-associated
     virus AAV2 expression vectors containing the α-galactosidase A gene
     fused to the liver-specific DC190 promoter were prepared  The DC190 promoter
```

consists of the human serum albumin promoter to which 2 copies of the human prothrombin enhancer were appended. Fabry mice administered this vector developed an immune tolerance to the enzyme.

=> d au ti so pi 1-4 l12

L12 ANSWER 1 OF 4 MEDLINE on STN DUPLICATE 1
AU Jacobs F; Snoeys J; Feng Y; Van Craeyveld E; Lievens J; Armentano D; Cheng S H; De Geest B
TI Direct comparison of hepatocyte-specific expression cassettes following adenoviral and nonviral hydrodynamic gene transfer.
SO Gene therapy, (2008 Apr) Vol. 15, No. 8, pp. 594-603. Electronic Publication: 2008-02-21.
Journal code: 9421525. E-ISSN: 1476-5462.

L12 ANSWER 2 OF 4 MEDLINE on STN DUPLICATE 2
AU Yasuda Makiko; Domaradzki Maciej E; Armentano Donna; Cheng Seng H; Bishop David F; Desnick Robert J
TI Acute intermittent porphyria: vector optimization for gene therapy.
SO The journal of gene medicine, (2007 Sep) Vol. 9, No. 9, pp. 806-11.
Journal code: 9815764. ISSN: 1099-498X.

L12 ANSWER 3 OF 4 CAPLUS COPYRIGHT 2008 ACS on STN
IN Cheng, Seng H.; Meeker, David
TI Combined enzyme replacement, gene therapy and small molecule therapy for lysosomal storage diseases
SO U.S. Pat. Appl. Publ., 35 pp., Cont.-in-part of U.S. Ser. No. 884,526.
CODEN: USXXCO

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI US 20040204379	A1	20041014	US 2004-758773	20040116
US 20020095135	A1	20020718	US 2001-884526	20010619
US 20070280925	A1	20071206	US 2007-762689	20070613

L12 ANSWER 4 OF 4 CAPLUS COPYRIGHT 2008 ACS on STN
IN Souza, David W.; Armentano, Donna; Wadsworth, Samuel C.
TI Combination of liver specific enhancer and strong promoter elements used in vectors for delivery to the liver
SO PCT Int. Appl., 22 pp.
CODEN: PIXXD2

PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI WO 2001036620	A2	20010525	WO 2000-US31444	20001115
WO 2001036620	A3	20020214		
W: AU, CA, JP				
RW: AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, TR				
EP 1232276	A2	20020821	EP 2000-982132	20001115
EP 1232276	B1	20070418		
R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO, MK, CY, AL, TR				
AT 360082	T	20070515	AT 2000-982132	20001115
EP 1818407	A2	20070815	EP 2007-7344	20001115
EP 1818407	A3	20070829		
R: AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LI, LU, MC, NL, PT, SE, TR				
ES 2284545	T3	20071116	ES 2000-982132	20001115
US 20030017139	A1	20030123	US 2002-139763	20020506
US 7312324	B2	20071225		

US 20080070297 A1 20080320 US 2007-938906 20071113

=>
=>